#### CDER 2016 Priorities

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### CDER has Multiple Important Priorities

- Diverse stakeholders: all have expectations that their priorities will be addressed (promptly!)
- Congress has provided ongoing priorities in Statutory form: FDAAA, FDASIA, DQSA, Sunscreen Innovation Act, appropriations bill language; more under consideration currently
- Operation of four user fee programs with multiple ongoing goal commitments
- All relate to underlying mission of ensuring an accessible supply of safe and effective drugs, and preventing introduction of unsafe, ineffective or counterfeit drugs

# HOW DID WE DO AGAINST LAST YEAR'S PRIORITIES?

#### 2015 Priorities: Front Burner

- GDUFA: meeting and exceeding performance goals; confident we can reach steady state in two years
- Stabilization of new Office of Generic Drugs: accomplished
- Standup of Office of Pharmaceutical Quality: completed
- Completion of 2015 PAG agreement work: done
- Pharmacy compounding:
  - Establishment of AC; multiple meetings
  - Multiple draft and final Guidances issued

### 2015 Priorities: Front Burner

- Standup of Panorama (new IT system for workflow management, document and data access): accomplished--generic drug review process running on Panorama
- Respond to Sunscreen Innovation Act: have met all milestones
- Abuse-Deterrent Opioids Final Guidance: Issued 4/15
- Respond to Congressional requests on "21st Century Cures" legislation: done

## 2015 Priorities: Important

- Established Sentinel Network in OSE: intent to utilize in routine drug safety activities
- OTC monograph reform: discussion of new approach with Congress and industry ongoing
- Sustainable model for ICH: completed, initial technical meeting held last week
- Biosimilars Program:
  - Issued draft "Nonproprietary Naming" guidance in 8/15
  - 1<sup>st</sup> biosimilar approval, for filgrastim

- Posting demographic information about newly approved drugs: "Drug Snapshots" program established
- Important guidances issued (many), for example:
  - Draft on Duchenne Muscular Dystrophy
  - Draft on Reporting Quality Metrics
- Build in-house OD capacity: New CDER leadership development program established
- GLP testing oversight function: established in OTS
- Management: implementation of LIRA, new budget management system

#### **2016 CDER PRIORITIES**

### Front Burner Priorities: 2016

- Negotiate PDUFA 6 agreements
- Negotiate GDUFA 2 agreements
- Negotiate a second BSUFA program
- Continue to implement new (and clarified) statutory provisions on drug compounding and outsourcing facilities
- Continue to meet milestones of Sunscreen Innovation Act
- Continue implementation of Track and Trace program
- Respond as needed and participate as requested in Congressional inquiries and FDA-related legislative initiatives

#### Front Burner Priorities

- Re-evaluate our regulation of drug advertising and promotion in light of current jurisprudence around the 1<sup>st</sup> Amendment: ongoing, progress made, but more work needed
- Continue to plan for and build out Panorama for new drug review process and other regulatory functions
- Prescription opioid epidemic:
  - Issue draft guidance on generic versions of abuse-deterrent opioid formulations
  - Evaluate opioid labels and REMS
  - Work on appropriate prescribing through our Safe Use group
- Improve staffing:
  - Continue to have more than 600 staff vacancies
  - Recruiting for multiple executive positions

# Important Priorities (in no order)

- Integrate Sentinel Network into routine drug safety activities
- Further implement biosimilars program, issuing additional guidance and launching educational efforts (Recent Senate hearing)
- Further implement statutory provisions related to the drug supply chain and "track and trace"
- Continue Drug Label Improvement Initiative
- Develop legal framework for Patient Medication Information (PMI) project

- Respond to outbreaks (ongoing)
- Develop a process and ultimate policy documents on evalution of a biomarker as a surrogate endpoint for accelerated approval
- Develop a strategic plan for managing drug imports: progress made
- Full staffing and process standardization in re-organized Office of Compliance
- Continue to refine policies around personalized medicine; ongoing
- Continue to develop policy approach for antimicrobials to treat drug-resistant organisms
- Establish well-documented OPQ operations

- Ongoing assessment of impact of "Breakthrough Therapies" program
- Further implementation of qualitative benefit/risk assessment
- Modernize clinical evidence development, fully utilizing electronic health data (priority of Dr. Califf)
- Further advance our patient-focused drug development program
- Evaluate the impact of requiring CV safety studies for certain chronic indications, e.g., diabetes and obesity (underway)

- Make significant progress on FDA-EU mutual reliance initiative
- Continue to push standards development and standardized electronic submissions
- Refine biomarkers qualification program
- Improve combination product Inter-Center review process

- Advance progress of the more than 22 consortia CDER is collaborating with (OTS lead)
- Work on ways to get drugs not supported by PREA/BPCA studied in children: new external pediatric network and neonatal consortium
- Develop implementation plan and training for pregnancy/lactation label rule
- Further develop use of Bayesian statistics, adaptive designs, modeling approaches, etc. for difficult drug evaluation issues
- Ones I can't talk about (because they are predecisional, under review, etc.)

## **Continuing Priorities**

- These have been previous high priorities and they are continuing to perform well:
  - PDUFA and GDUFA processes: meeting the goals
  - FOI: Reducing the backlog in the face of a higher request rate
  - Advisors and Consultants: holding AC meetings
  - OSE operations: multiple safety functions
  - CDER research functions: well-organized,
    integrated with regulatory staff, and productive

# Important Administrative/Managerial Priorities

- Evaluation of CDER governance system: improvements underway
- Develop a more mature quality management system
- Implement CDER Diversity Plan
- Plan and implement a new time reporting system
- Continue to look at root causes for Employee Viewpoint Survey Results lowest scores (CDER generally gets excellent scores overall in this survey)

# FURTHER INFORMATION ON SELECTED PRIORITIES

### **GDUFA Goals**

- CDER has met GUDFA goals so far
- Action date goals went into effect 10/1/14 for newly filed applications; now on second cohort (10/1/15) with goals
- These are being managed in new IT system and are all on track
- One significant goal is to minimize multiple application cycles: information requests to sponsor during review process
- Choke points in review process identified and being dealt with; although some of these will take time (inspections)

# Standup of OPQ "Super-Office"

- Office formally stood up Jan, 2015
- Recruitment of Office Director—Michael Kopcha
- Implementation of "Integrated Quality Assessment"=team review
- Construction of facilities inventory in Office of Surveillance and issuance of "work plan"
- "New inspection protocol" pilot with ORA

# New Surveillance Function: Quality Metrics

- Proposed (draft guidance) to collect wellunderstood metrics from facilities regarding state of quality: evaluating comments
- Metrics widely used in quality management in most large-scale manufacturing sectors
- Often combined in "dashboard" to alert management to impending problems
- Takes time to understand metrics and make sure they represent the same measure across various groups; pilots ongoing

### CDER-ORA PAG Agreement

- Integrate ORA facility pre-approval inspections into OPQ team review—one overall quality assessment. Pilots ongoing; new inspectional template under development
- Specialized pharmaceutical inspectional personnel in ORA will work closely w Center; ORA undergoing change process
- Share data from various systems seamlessly
- CDER hopes to provide tablets or other handhelds to streamline report generation

## **New IT System**

- Workflow management, data and document management components
- Plan to implement in all Center processes over multi-year process
- "Pharmaceutical Platform" has begun to instantiate wish list of quality reviewers. Has already replaced multiple non-connected databases
- Vast need of Center programs to have integrated, standardized work platform; multi-year plan to get there

### "Personalized Medicine" Policies

- CDER is approving significant number of "targeted therapies"
- These drugs target pathways or specific genetic mutations and thus are less disease-specific
- Target populations tend to be narrow sub-populations of specific diseases; and developers then seek to get additional indications
- Efficacy requirements for these additional "small slices" are under consideration. Have used case-by case evaluation up to now, but broader policy development is needed
- Workshop was held 12/12/14 at White Oak on this topic

# Streamlining Clinical Trials: Multiple Projects Ongoing

- Work with stakeholders on:
  - "Basket trials" and "master protocols"
  - Randomized clinical trials embedded in healthcare system
- Use of new IT
  - Use of personal devices for patient input
  - Use of telemedicine in clinical trials
- "Monitoring and Data Cleaning Practices":
  - Traditional monitoring may not be most effective way of ensuring data quality: building quality in; developing riskbased approaches, and focusing on the most important data points may provide better quality

# Evaluation of Breakthrough Therapy Designation Program

- Pace of submissions and designations continue
- Initial evaluation of 1<sup>st</sup> two years conducted by OSP
- Surveyed medical staff; did not survey industry
- Have instituted streamlined approach to triage applications for designation
- Industry input will be helpful in determining the value of the program: did it help and, if so, how was the designation helpful? Evaluation will be done under contract.

### "Patient-focused" Drug Development

- We understand that people with chronic diseases are "experts" in that disease, as far as the symptoms and the impact on QOL, and what might be acceptable tradeoffs
  - On risk
  - On uncertainty
- How to meaningfully collect that knowledge, in rigorous manner, given that there is a spectrum of opinions and and a spectrum of disease burden in any given disease?
- How to do this for the many thousands of diseases?
- Working with multiple patient organizations who are pioneering patient-focused guidance development for their disease of focus

# Importance of Good Management

- In addition to these priority initiatives and other initiatives, CDER has a large volume of work that must be accomplished every day: we are a production shop
- Tens of thousands of decisions made yearly on INDs, applications and supplements; thousands of meetings with industry; more than 50 guidances and multiple regulations published; FOI work; AC's; import decisions; drug safety communications; underlying drug safety evaluation activities; evaluation of inspection results; compliance and enforcement actions; responses to citizen petitions and lawsuits, and scientific activities, to name just a few.
- Ensuring that all this gets done, well and efficiently, requires engaged staff members who feel supported and listened to by leadership, careful process and quality management, and highquality IT support

# Importance of Good Management

- Many of our stakeholders have policy priorities and do not understand how critical good management is to making things happen; seems to be a general problem in government
- It is feasible to handle a handful of initiatives through an informal process, but not hundreds, while at the same time managing the ongoing workload
- CDER's "lean team" assists with process improvement throughout the Center
- We have a plan for implementing modern IT process and data support: accomplishing these longer-term goals will be key to sustaining our success

## Summary

- CDER has numerous priority initiatives for 2016 along with ongoing workload
- Outstanding progress has been made in many areas, but we are all quite pressed
- Large number of staff vacancies also require VERY significant amount of work to fill
- Attention to continuous improvement in management and IT support will enable accomplishment of a broad agenda